



On approval rules for formation of the list of orphan diseases and medicines for their treatment

Unofficial translation

Order of the Minister of Healthcare of the Republic of Kazakhstan dated October 16, 2020 № ҚР ДСМ-135/2020. Registered with the Ministry of Justice on October 19, 2020 № 21454.

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In accordance with clause 4 of article 177 of the Code of the Republic of Kazakhstan dated July 7, 2020 "On public health and Health Care System", **I HEREBY ORDER:**

1. To approve the Rules for formation of the list of orphan diseases and medicines for their treatment according to the Appendix to this order.

2. The Department for Organization of Medical Care of the Ministry of Health of the Republic of Kazakhstan, in accordance with the procedure, established by law, shall ensure:

1) state registration of this order with the Ministry of Justice of the Republic of Kazakhstan;

2) placement of this order on the Internet resource of the Ministry of Healthcare of the Republic of Kazakhstan after its official publication;

3) within ten working days after the state registration of this order, submission to the Legal Department of the Ministry of Healthcare of the Republic of Kazakhstan, of information about implementation of activities, stipulated by subclauses 1), 2) of this clause.

3. Control over execution of this order shall be entrusted to the supervising Vice-Minister of Healthcare of the Republic of Kazakhstan.

4. This order shall come into force upon expiry of ten calendar days after the date of its first official publication.

*Minister of Healthcare
of the Republic of Kazakhstan*

A. Tsoy

Appendix
to the to the Order of the
Minister of Healthcare of the
Republic of Kazakhstan
dated October 16, 2020
№ ҚР ДСМ-135/2020

Rules for formation of the list of orphan diseases and medicines for their treatment Chapter 1. General Provisions

1. These Rules for formation of the list of orphan diseases and medicines for their treatment (hereinafter referred to as the Rules) have been developed in accordance with clause 4 of article 177 of the Code of the Republic of Kazakhstan dated July 7, 2020 "On public

health and Health Care System" (hereinafter referred to as the Code) and shall determine the procedure for the formation of the list of orphan diseases and medicines for their treatment.

2. The following concepts are used in these Rules:

1) scientific organization in the field of public health - a national center, scientific center or research institute carrying out scientific, scientific and technical and innovative activities in the field of healthcare, as well as medical, pharmaceutical and (or) educational activities;

2) authorized body in healthcare (hereinafter referred to as the authorized body) – a central executive body that carries out management and cross-sectoral coordination in the field of health protection of citizens of the Republic of Kazakhstan, medical and pharmaceutical science, medical and pharmaceutical education, sanitary and epidemiological well-being of the population, circulation of pharmaceuticals and medical devices, the quality of healthcare services (assistance);

3) orphan (rare) diseases – rare severe diseases that threaten human life or lead to disability, the frequency of which does not exceed the officially defined level;

Chapter 2. Procedure for formation of the list of orphan diseases

3. To include a disease into the list of orphan diseases, healthcare entities, representatives of associations and public associations, individuals and legal entities shall submit proposals to the authorized body.

4. Recommendations from the moment of receipt within 3 working days are sent by the authorized body to scientific organizations in the field of health care according to the profile of the disease and (or) to a scientific organization in the field of health care that promotes the development of health care through research, implementation of scientific, technical, information and educational programs (hereinafter referred to as the Working Body).

Footnote. Paragraph 4 - as amended by the order of the acting Minister of Health of the Republic of Kazakhstan dated 31.12.2021 № KR DSM-142 (shall be enforced ten calendar days after the day of its first official publication).

5. In scientific organizations in the field of health care, recommendations to include a disease in the list of orphan diseases are considered by a commission.

The commission shall be created by order of the head of a scientific organization in the field of health, which includes the chairperson, members of the commission (at least three specialized specialists, including freelance health professionals) and a secretary.

The chairperson is elected from the members of the commission by voting.

Footnote. Paragraph 5 as amended by the order of the acting Minister of Health of the Republic of Kazakhstan dated 31.12.2021 № KR DSM-142 (shall be enforced ten calendar days after the day of its first official publication).

6. The commission on proposed diseases shall conduct an analysis that includes the following information:

1) general description of the disease or condition;

2) the code in accordance with the International Classification of Diseases (hereinafter referred to as the ICD);

3) description of etiology and symptoms;

4) the prevalence of orphan disease, calculated on the basis of statistical data of the Republic of Kazakhstan and (or) international statistical data;

5) justification of the severity or disabling nature of the disease based on objective and quantitative medical or epidemiological information;

6) justification for the life-threatening nature of the disease or condition based on mortality rates and life expectancy.

7. The conclusion of the commission shall be drawn up in a protocol, which contains the name of the medical organization, the date of the meeting, the list of members present, the list of issues discussed, the decision and its justification.

The conclusion of the commission shall be sent to the authorized body for consideration at the meeting of the Joint Commission on the quality of healthcare services, formed in accordance with article 15 of the Code (hereinafter referred to as the JCQ).

8. The authorized body shall make a decision on inclusion of a disease to the list of orphan diseases if there are positive recommendations of the JCQ and compliance with the subclause 1) and one of subclauses 2), 3), 4), 5) of the following criteria:

1) prevalence in the Republic of Kazakhstan;

2) the need for systematic treatment, for which there are medicinal products developed and registered in the Republic of Kazakhstan (curable patients);

3) the need in systematic treatment with the use of drugs that are developed and available in the world, but are not registered for use on the territory of the Republic of Kazakhstan;

4) the presence of diseases for the treatment of which there are no developed medicines in the world;

5) the need in provision of palliative care due to the lack of radical therapy (incurable patients).

Prevalence in the Republic of Kazakhstan is no more than 50 cases per 100 thousand of population in the Republic of Kazakhstan.

9. Revision of the list of orphan diseases shall be carried out once in three years and (or) upon occurrence of a new reliable case.

Chapter 3. Procedure for formation of the list of medicines for the treatment of orphan diseases

10. To include medicines to the list of orphan medicines, the entities of circulation of medicines, representatives of manufacturers of medicines, healthcare entities, representatives of associations and public associations, individuals and legal entities shall submit proposals to the authorized body.

11. Recommendations from the moment of receipt within 3 working days are sent by the authorized body to the Working Body.

Footnote. Paragraph 11 - as amended by the order of the acting Minister of Health of the Republic of Kazakhstan dated 31.12.2021 № KR DSM-142 (shall be enforced ten calendar days after the day of its first official publication).

12. The Working body shall carry out the analysis of proposed medicines with preparation of a conclusion, which includes the following information:

- 1) general description of the medicine;
- 2) information about the registration of a medicine in the territory of the Republic of Kazakhstan with the status of an orphan medicine (technology) or description of reasons of the absence of registration in the Republic of Kazakhstan;
- 3) general description of an orphan disease, included into the List of orphan diseases, approved in accordance with clause 3 of article 177 of the Code or a narrower indication within the framework of this orphan disease, in which the use of medicine is proposed;
- 4) details of the regulatory orphan status abroad;
- 5) overview of major clinical trials;
- 6) comparison with orphan technologies registered in the Republic of Kazakhstan with confirmation of significant clinical or economic benefits.

13. The results of the analysis in the form of a conclusion of the Working Body shall be sent to the authorized body for consideration at a meeting of the Formulary Commission (hereinafter referred to as the FC), determined in the manner prescribed by paragraph 2 of Article 264 of the Code.

14. A medicine shall be included into the List of Orphan Medicines if there is a positive FC recommendation and the following criteria are met:

- 1) the medicine is intended for the diagnosis, prevention or treatment of one or more orphan diseases according to the approved list of orphan diseases (at the time of submission of the proposal);
- 2) the medicine is registered for the use in the territory of the Republic of Kazakhstan with a status of an orphan medicine (technology) or not registered for the use in the territory of the Republic of Kazakhstan;
- 3) the medicine is intended for patients with a disease, for which there are no satisfactory methods of diagnosis, prevention, treatment, or if such a method is available, the medicine will be of significantly greater benefit to patients suffering from this disease.

15. Revision of the List of orphan medicines shall be carried out once in a three year and (or) upon occurrence of new orphan medicines.